

**CIRM Scientific and Medical Research Funding Working Group
Biographical information of candidates nominated to serve as
Alternate Scientific Members of the Working Group**

Laurie Jackson-Grusby, Ph.D.

Dr. Jackson-Grusby is an Assistant Professor at Children's Hospital Boston and Harvard Medical School Departments of Pathology, Principal faculty at Harvard Stem Cell Institute, and Affiliate faculty member of the Kirby Center for Neuroscience at Children's Hospital. She earned her Ph.D. in Genetics working with Dr. Philip Leder at Harvard Medical School on the molecular embryology of limb deformity and characterization of formin proteins. She worked as a postdoctoral fellow with Dr. Rudolf Jaenisch at the Whitehead Institute and MIT, publishing several mechanism directed studies on cancer epigenetics and the first functional epigenomics study of the DNA methylome.

Dr. Jackson-Grusby's current research focus is on cancer stem cells and epigenetic mechanisms that maintain proper homeostasis of stem and progenitor cells. Her lab has implemented quantitative methylation screens using bisulfite-mass spectrometry to characterize aberrant development and cancers arising from pure epimutations. They have developed new animal models for deciphering the stem cell basis of medulloblastoma, a pediatric brain tumor. The Jackson-Grusby group develops and utilizes stem cell applications for fractionation and genetic analysis of cancer stem cells in brain tumors as a means to identify new therapeutic concepts for treatment refractory tumors.

Dr. Jackson-Grusby is a member of the NIH External Steering Committee for the Roadmap Epigenome Centers. She serves on the editorial board of *Epigenomics*, and provides stem cell and epigenetics expertise to numerous NIH review panels. Dr. Jackson-Grusby is a recipient of a Distinguished Scientist Award from the Sontag Foundation and is an Elinor and Miles Shore Scholar at Harvard Medical School.

Maria Grazia Roncarolo, M.D.

Maria Grazia Roncarolo is the Scientific Director of the San Raffaele Scientific Institute in Milan, Italy, and Professor in Pediatrics, School of Medicine and Surgery, San Raffaele Vita-Salute University. She earned her M.D. at the University of Turin, specialized in Pediatrics and Immunology. Dr. Roncarolo worked in Lyon for several years at the Edouard Herriot Hospital and at the Laboratory for Immunological Research UNICET on the mechanism of tolerance in severe combined immunodeficiency (SCID) patients transplanted with allogenic hematopoietic stem cells (HSCs). She worked for more than 8 years at the DNAX Research Institute of Molecular and Cellular Biology, Human Immunology Department, in Palo Alto, CA, on the basic biology of HSCs, cytokines and transplantation tolerance. Since 1998 she has been working at San Raffaele Telethon

Institute for Gene Therapy (HSR-TIGET), of which she has been Director from 2000 until September 2008. Since July 2003, she is Chief of Clinic, Pediatric Immunology and Hematology and Clinical Research Unit (CRU-P), San Raffaele Hospital and San Raffaele Scientific Institute.

Dr. Roncarolo has a long-lasting interest in the mechanisms that induce and maintain tolerance in bone marrow transplantation (BMT), organ transplantation, autoimmune disease and gene therapy. Her research focuses on the mechanisms underlying T-cell tolerance, anergy and activation. She was among the first to convincingly prove that active suppression is mediated by regulatory T cells, and her group identified and biologically characterized a new subset of regulatory T cells named T regulatory type 1; this work was published in *Nature*. It has since been demonstrated by several groups, including that of Dr. Roncarolo, that T regulatory type 1 cells play a key role in immunological homeostasis and in prevention of autoimmune diseases. In addition, Dr. Roncarolo is interested in investigating the mechanisms underlying the immune defects, and identifying new cures for children with SCID and other forms of primary immunodeficiencies. Her work includes gene transduction of HSCs and gene therapy in primary immunodeficiencies and metabolic diseases. Dr. Roncarolo's clinical interests include primary immunodeficiencies, autoimmune diseases, allogenic BMT, gene therapy clinical trials, and hemoglobinopathies.

Dr. Roncarolo is member of the Academia Europaea of Sciences and has been awarded the honor of Ufficiale dell'Ordine "Al Merito della Repubblica Italiana" for scientific merits. She is among the world's most cited scientists according to ISIHighlyCited.com (Thomson Reuters, Philadelphia). Overall, her papers have been cited over 9800 times. Dr. Roncarolo has published more than 225 papers in international scientific peer review journals with a total impact factor of 2222 and an average impact factor of 9.80 and has contributed 22 chapters to books. Dr. Roncarolo is the inventor of 11 international patents issued or pending.